

Fig. 1

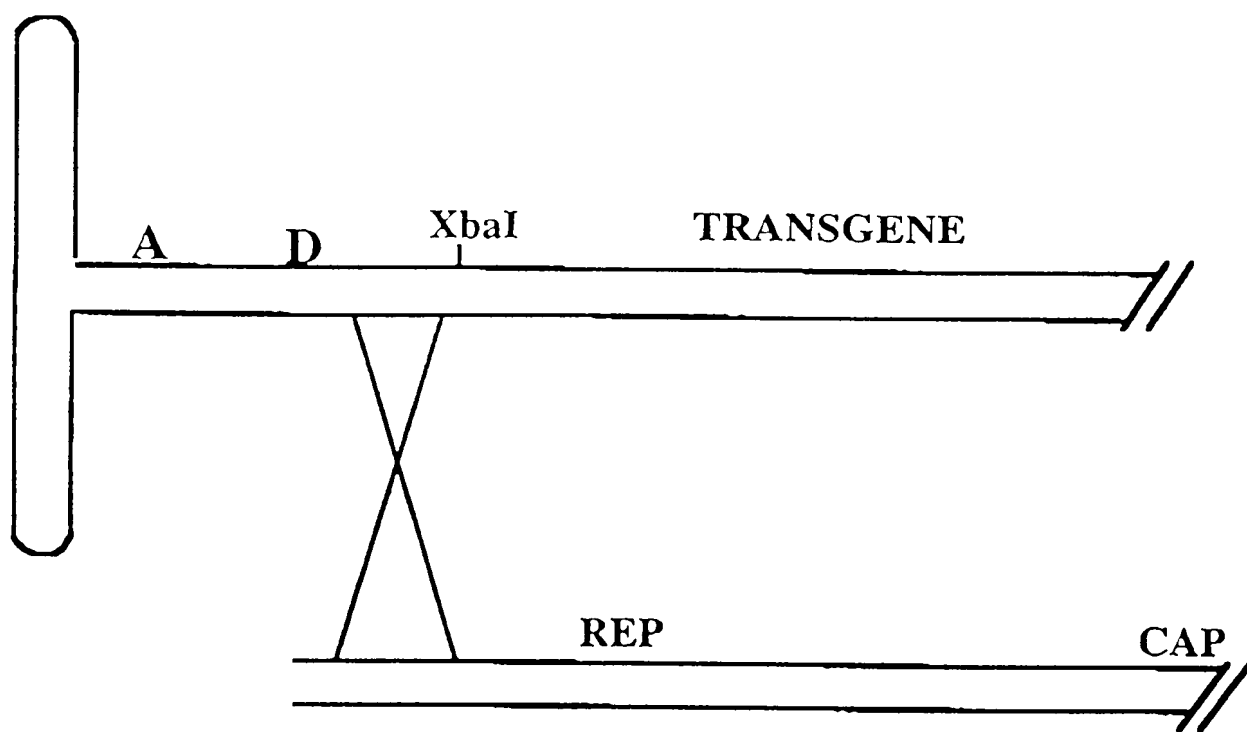
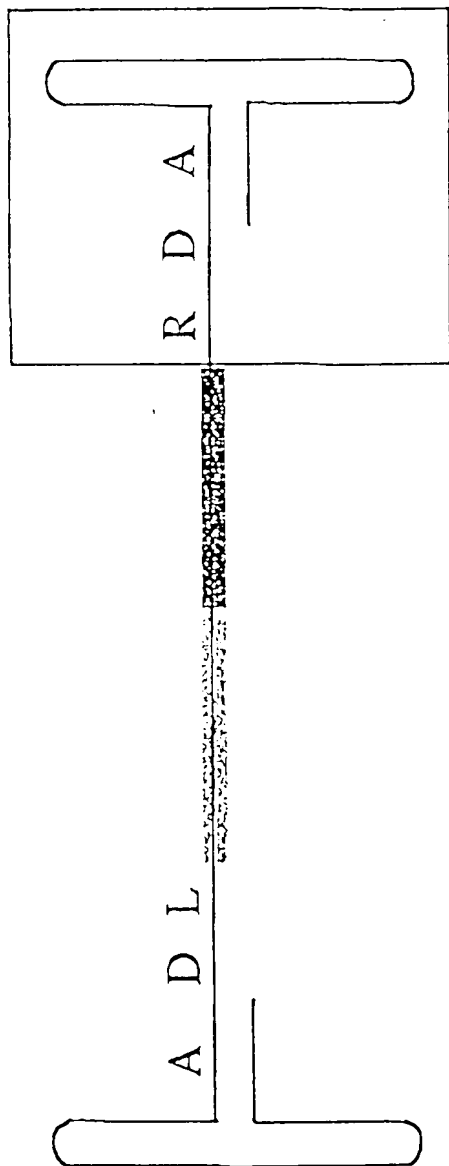


Fig. 2

wild-type



pSub201

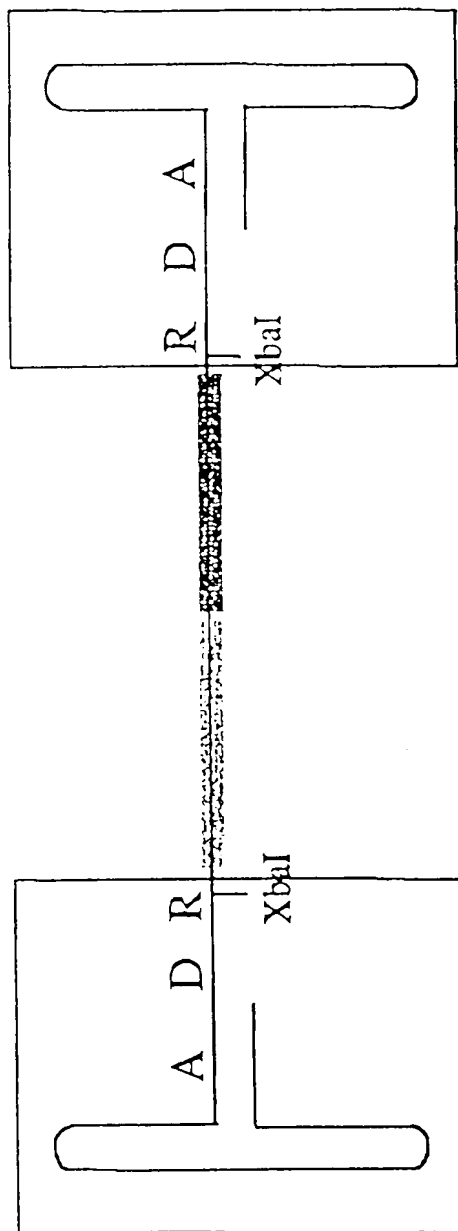


Fig. 3

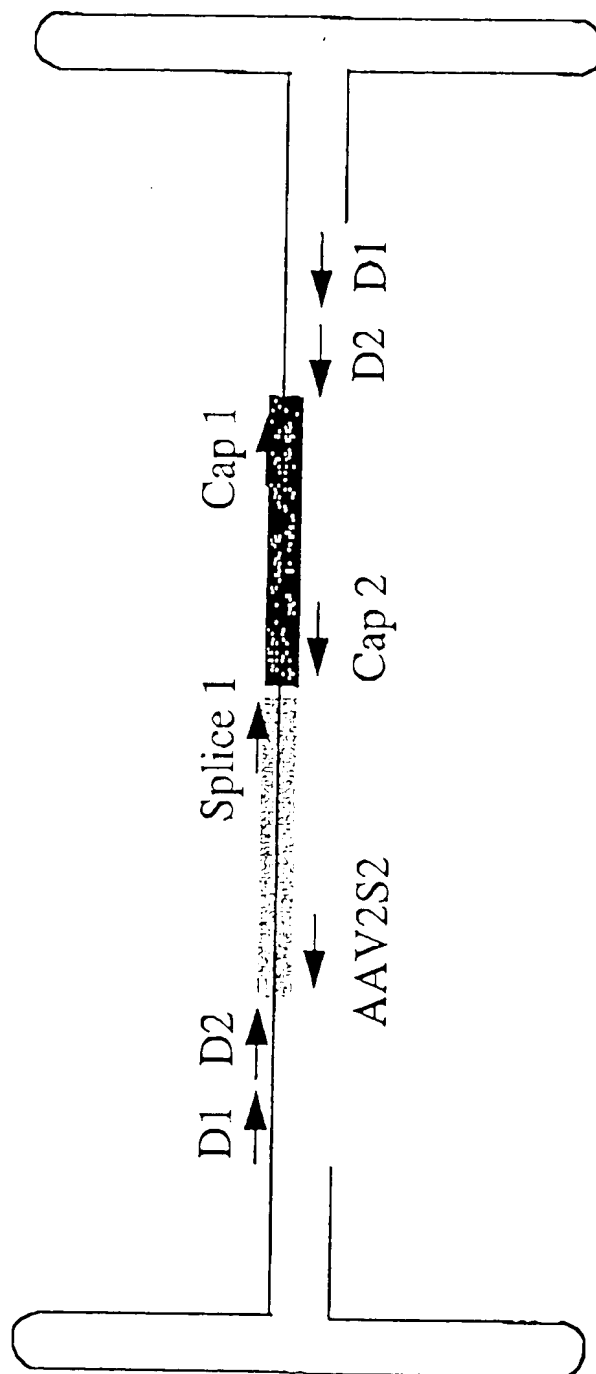


Fig. 4

Wild-Type

AGAGGGAGTGGCCAA CTCCATCACTAGGGGTTCCCT GGAGGGGTGGAGTCGTGACGTGAATTACGTCATAGGGTTAGGGAGGTCC

A D L

pSub201

AGAGGGAGTGGCCAA CTCCATCACTAGGGGTTCCCT TGTAGTTAATGATTAAACCCGCCCATGCTACTTATCTACGTAGCCATGCTCTAGA

A D R

Fig. 5

MEG → Human FIX
b6 b7C b7D
b6 b7C b7D

Fig. 6

rAAV Production

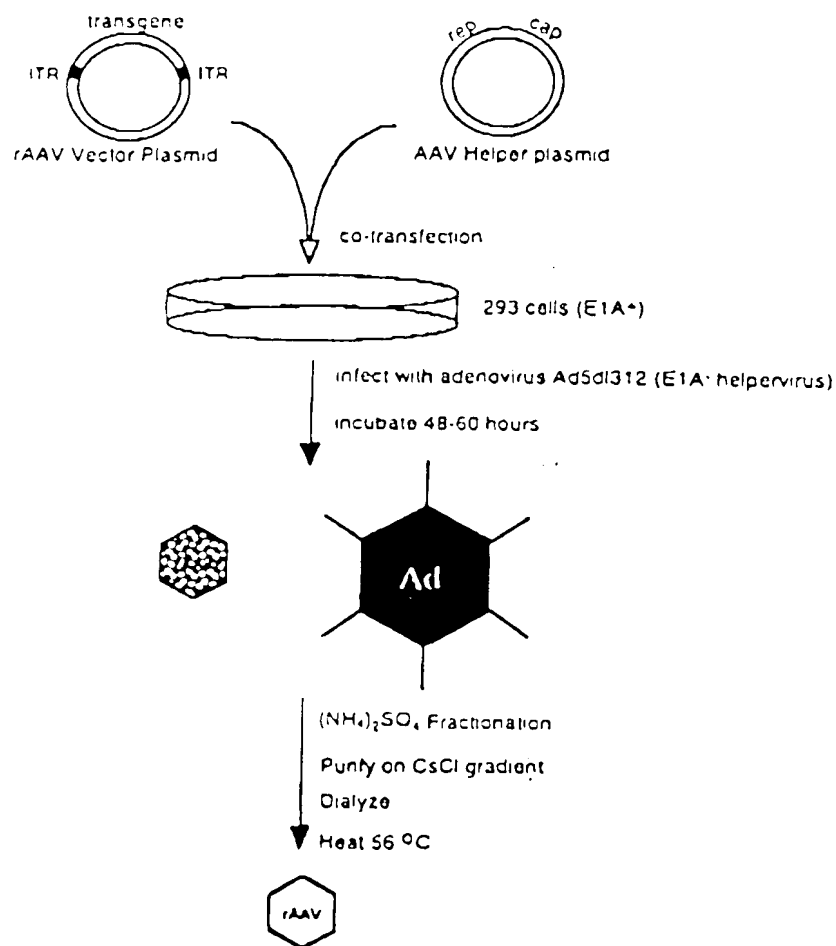
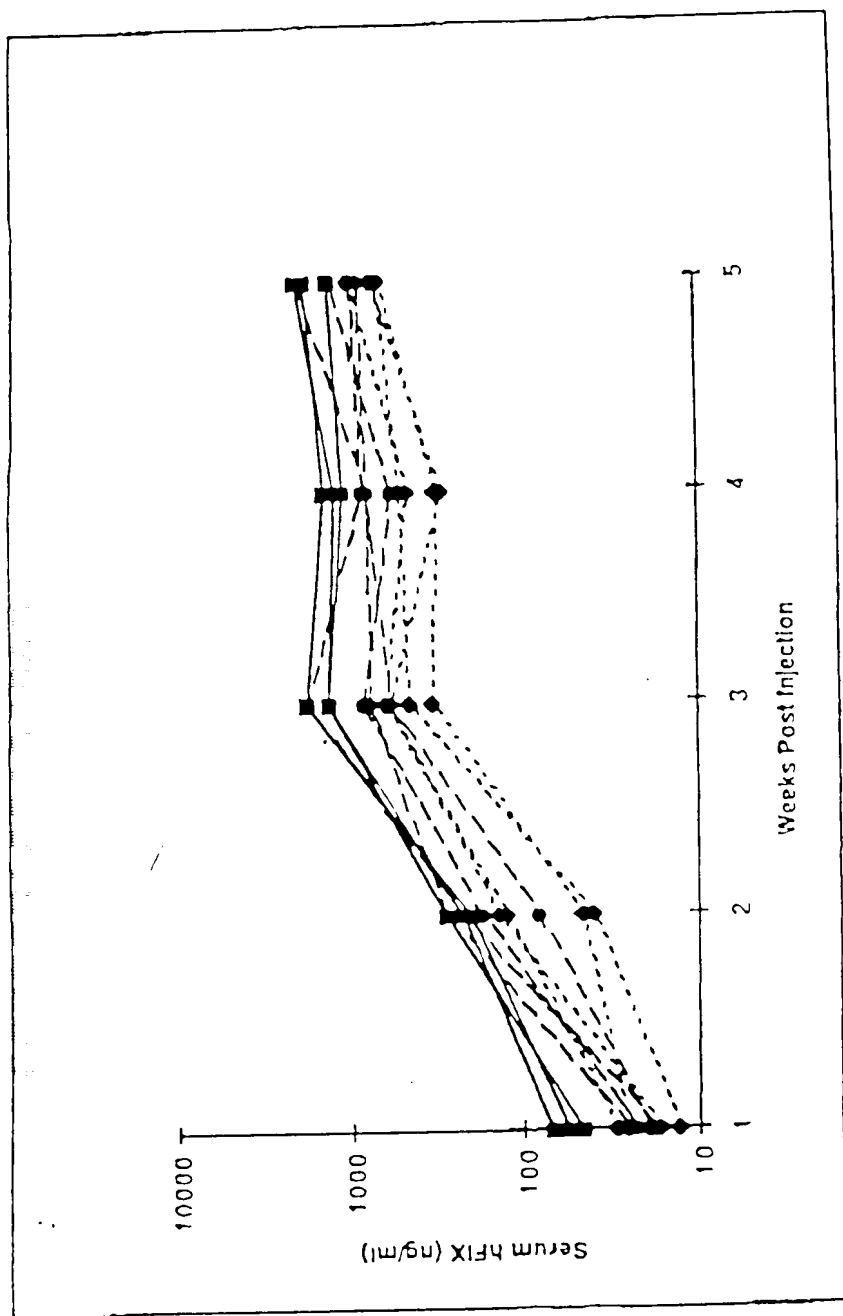
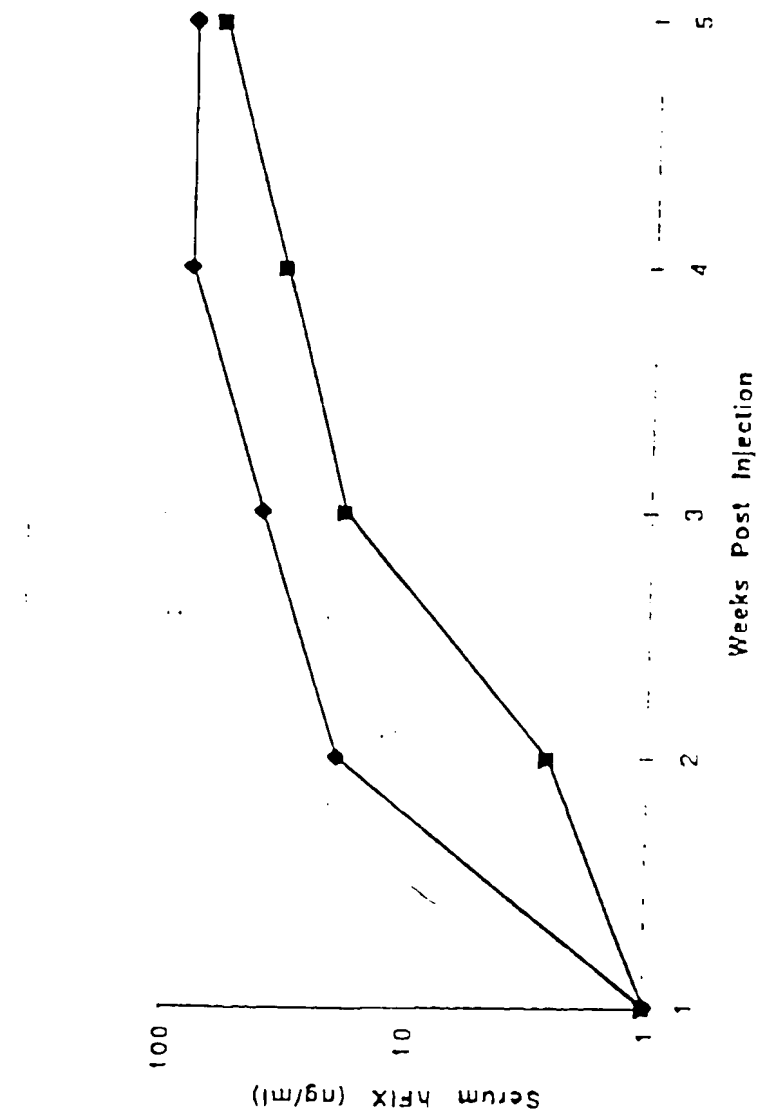


Fig. 7



---◆--- 2.1X10¹⁰ Particles
 ---◆--- 2.1X10¹⁰ Particles
 ---◆--- 2.1X10¹⁰ Particles
 ---◆--- 2.1X10¹⁰ Particles
 ---◆--- 2.1X10¹⁰ Particles
 ---●--- 4.2X10¹⁰ Particles
 ---●--- 4.2X10¹⁰ Particles
 ---●--- 4.2X10¹⁰ Particles
 ---●--- 4.2X10¹⁰ Particles
 ---●--- 4.2X10¹⁰ Particles
 ---■--- 8.4X10¹⁰ Particles
 ---■--- 8.4X10¹⁰ Particles
 ---■--- 8.4X10¹⁰ Particles

Fig. 8



— 4.2X10¹⁰ Particles (n=2)

Fig. 9

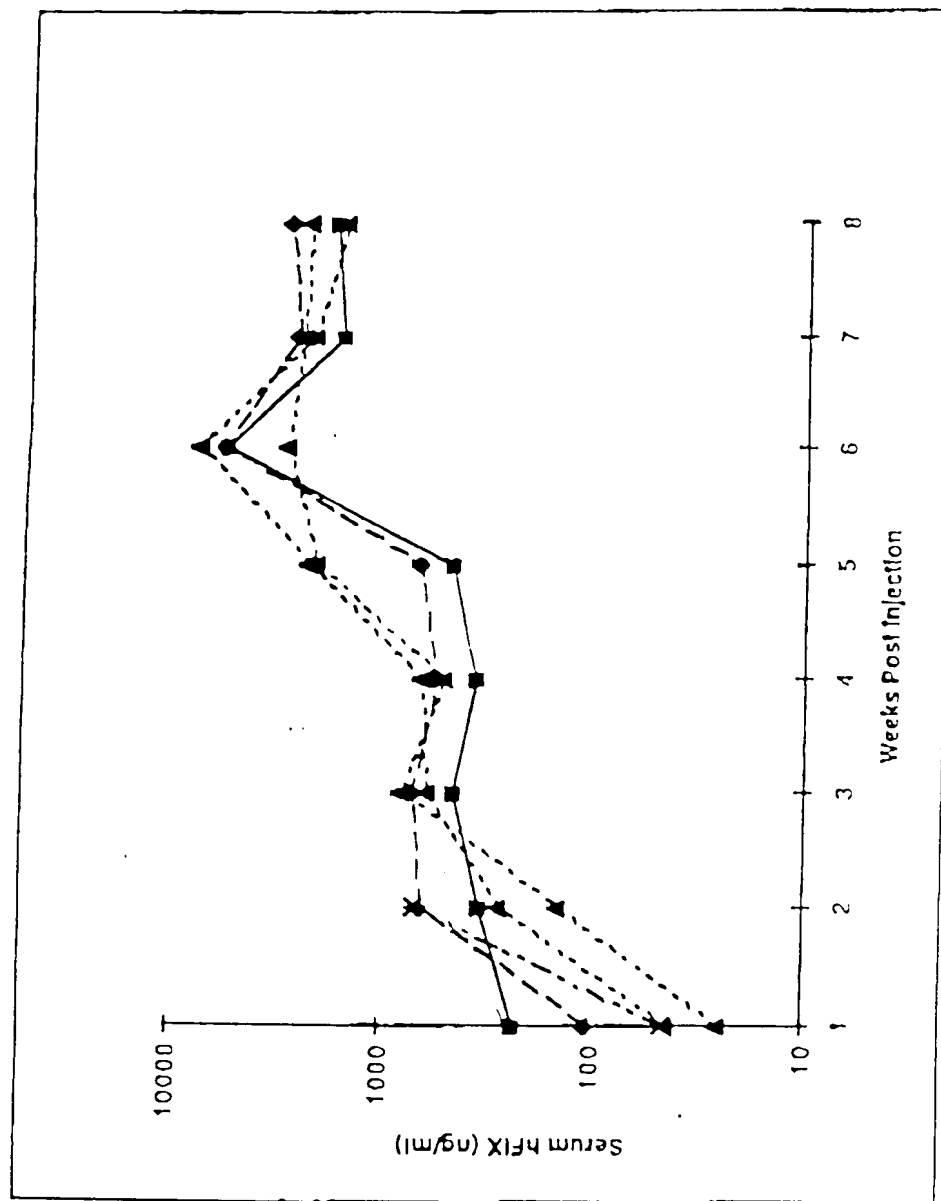


Fig. 10

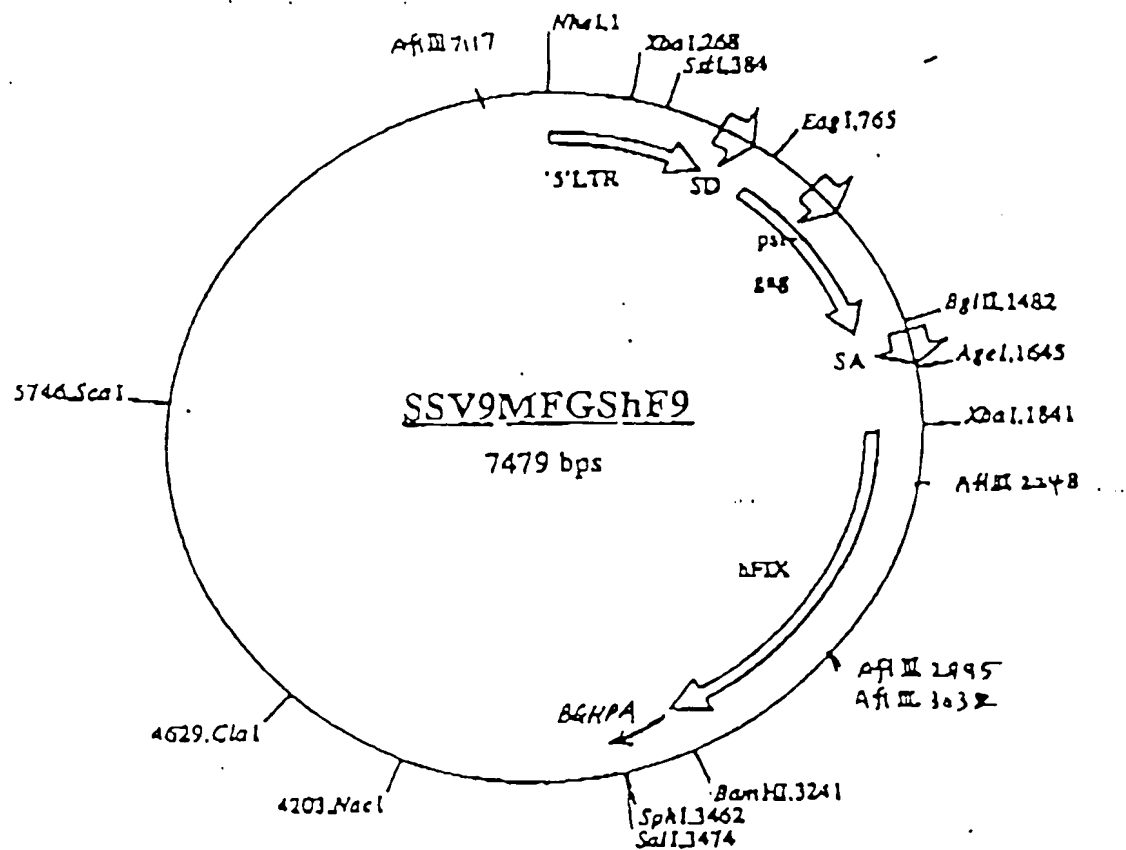


Fig. 11